CIRM Funded Clinical Trials

In Utero Hematopoietic Stem Cell Transplantation For The Treatment Of Fetuses With Alpha Thalassemia Major

Disease Area:	Fetal Alpha Thalassemia Major
Investigator:	Tippi MacKenzie
Institution:	University of California, San Francisco
CIRM Grant:	CLIN2-09183
Award Value:	$10,906,978
Trial Sponsor:	University of California, San Francisco
Trial Stage:	Phase 1
Trial Status:	Recruiting
Targeted Enrollment:	10
ClinicalTrials.gov ID:	NCT02986698

Tippi MacKenzie

Details:
Dr. MacKenzie and her team at UCSF are using hematopoietic stem cells (HSCs) to treat babies in the womb who have alpha thalassemia major, a blood disorder that is almost always fatal. Current treatment requires in utero blood transfusions and monthly blood transfusions for life or a bone marrow transplant if a suitable donor is identified. In this trial, HSCs are taken from the mother's bone marrow and transplanted into the baby before birth. The mother's cells are able to survive and correct the defect in the baby's blood cells, increasing the chances of a healthy birth and improving the chances of having effective treatments after birth.

Design:
Single arm, non-randomised study.

Goal:
Safety and feasibility, efficacy.

Updates:
This trial is currently recruiting participants.

News Releases:
How a UCSF study could change the lives of babies in the womb
Stem Cell Agency invests more than $44 million in treatments for stroke and fixing blood disorders in the womb

Contact Trial Sponsor

Source URL: https://www.cirm.ca.gov/clinical-trial/utero-hematopoietic-stem-cell-transplantation-treatment-fetuses-alpha-thalassemia